

Thank you for joining the weekly webinar!

We are admitting audience members from the waiting room.

Please allow a few moments for the webinar to begin.



HEALEY ALS Platform Trial

Weekly Q&A – July 13, 2023



Healey & AMG Center

Sean M. Healey & AMG Center for ALS
at Massachusetts General Hospital





ALS Clinic & Research Program at OSU

Sarah Heintzman,
MS, APRN-CNP, FNP-C, CCRC
Neuromuscular Division

July 13th, 2023



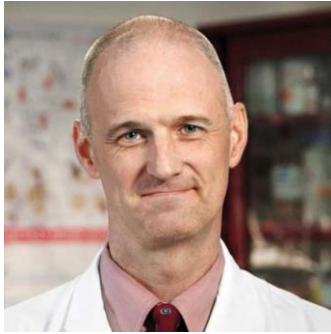
THE OHIO STATE UNIVERSITY
WEXNER MEDICAL CENTER

About Me

- 11 years of experience working in neuromuscular neurology at OSU currently as a Nurse Practitioner
- 17 years of experience working in clinical research in the following roles:
 - Clinical research coordinator
 - Senior research coordinator
 - Research nurse
 - Research manager
 - Sub-investigator
 - Principal investigator
- Co-chair of the HEALEY ALS Platform Trial Recruitment and Retention Committee, a member of the Cure SMA Care Center Network Committee, and is the Clinic Coordinator for the MDA and ALS clinics at OSU
- Certified clinical research coordinator as recognized by the Association of Clinical Research Professionals
- Spare time:
 - SCUBA, swimming, snorkeling
 - Mom to my 9 year-old son, Sam
 - Gardening, reading, travel & playing with my dog, Petey



The Team at OSU



Stephen J. Kolb, MD,
PhD; ALS/MND
Program Director



Adam Quick, MD



Jennifer Roggenbuck,
LGC



Cheyenne Grove, ALS
Research Program
Senior Coordinator

- Respiratory Therapy: Jerry Reynolds
- Social Work: Kinsey Ludt
- Occupational Therapy: Theresa Berner
- Physical Therapy: Andrea Jaworek
- Clinic Nurse: Alison Potter
- Registered Dietician: Kathryn Jira
- Speech Language Pathologist: Audrey Hall
- Research Coordination: Kaneshia Hives



Integrating Research into the Clinic

- Would you like to have a conversation about research?
- Knowledge is power
 - What is a clinical trial?
 - How do clinical trials help?
 - What is it like to be in a clinical trial?
 - How do I sign up for a clinical trial?
 - What if I decide I want to quit being on a clinical trial?
 - Who pays for a clinical trial?
- Discuss open studies at OSU
- Discuss the science - where are things headed?



Integrating Research into the Clinic

Smart Phrases in Epic

- Information can be easily updated
- Cost effective – no formal printing required
- Is printed out in the after visit summary

ALS Clinical Trials

The doctors, nurses, and researcher coordinators in Neuromuscular Neurology are committed to research, education, and patient care with the goal of finding effective new therapies for ALS. When you are a patient at the ALS multidisciplinary clinic, you have access to the newest treatments available. You may also have the opportunity to participate in a clinical trial. Clinical trials can help find new and better ways to prevent and treat ALS.

What is a clinical trial?

A clinical trial is a research study that follows a specific set of rules (protocol). This research includes using newer, promising treatments and the best methods currently available. Clinical trials help find ways to prevent, screen or treat ALS.

How do clinical trials help?

Taking part in a clinical trial may help you and may also help improve treatment for patients in the future. Being part of a clinical trial may give you early access to new drugs and treatments. When people participate in a clinical trial, it may help us find new treatment methods sooner.

What is it like to be in a clinical trial?

- You will be given detailed information about the clinical trial to help you decide if you want to be a part of the trial. A member of the health care team will explain why the trial is being done and what to expect. You and your family will have the opportunity to discuss your needs and have your questions answered. If you decide to participate, you will be asked to sign an informed consent form.
- It is important for you to follow the clinical trial protocol to have accurate results. The protocol will describe
 - Exactly how the clinical trial will be done
 - Who may sign up
 - What treatment is used
 - How the treatment is given
- How often you should be checked to evaluate the treatment
- A Clinical Research Coordinator will help coordinate your treatment, follow-up visits and tests to make sure they follow the protocol.

How do I sign up for a clinical trial?

Talk with a member of your health care team if you are interested in participating in a clinical trial. If your situation matches with a clinical trial that is currently available, you may be eligible to take part.

What if I decide I want to quit being on a clinical trial?

If you decide that you no longer want to take part in a clinical trial, you can stop at any time. If a person leaves a trial, they should talk to their doctor about other treatment options.

Who pays for a clinical trial?

Generally, there are no costs associated with participation in a clinical trial. You and your caregiver may also receive a stipend for your time. The stipend varies by trial so be sure

to ask the Study Coordinator whether or not the trial you are participating in provides a stipend.

What clinical trials are recruiting?

Currently Recruiting Trials at Ohio State

- Study of soluble factors in the cerebral spinal fluid in individuals with motor neuron disease
- Skin Fibroblast Isolation for the Derivation of Induced-Pluripotent Stem Cells for Tissue Culture Models of Neurological Disease
- MDA Registry Study - This study provides a way for researchers to follow the course of a disease
- A Phase 1-3 Study to Evaluate the Efficacy, Safety, Pharmacokinetics and Pharmacodynamics of Intrathecally Administered ION363 in Amyotrophic Lateral Sclerosis Patients With Fused in Sarcoma Mutations (FUS-ALS) - <https://www.ionispharma.com/medicines/ion363/>
- ALS Healey Platform Trial - <https://vimeo.com/424619821/53ed6c07a3>
 - <https://www.massgeneral.org/neurology/als/research/platform-trial>
 - Register for informative weekly webinars: [ALS Webinars and Symposiums \(massgeneral.org\)](#)

Other currently Recruiting Trials:

- COVID-19 ALS Registry, please visit - <https://redcap.link/COVID19ALSRegistrySurvey>
- ALS CDC Registry - <https://www.cdc.gov/als/ALSJoinALSRegistry.html>

Recently Completed Trials

- A Phase 3, Double-Blind, Randomized, Placebo-Controlled, Parallel Group, Multicenter Study With an Open-Label Extension to Evaluate the Efficacy and Safety of [Ravulizumab](#) in Patients With Amyotrophic Lateral Sclerosis (ALS)
- Phase III, Randomized, Double-Blind, Placebo-Controlled, Multicenter Trial to Evaluate the Safety and Efficacy of AMX0035 Versus Placebo for 48-week Treatment of Adult Patients with Amyotrophic Lateral Sclerosis (ALS)

To find more information about ALS clinical trials:

- Talk to your provider and/or members of your treatment team. Contact Cheyenne Grove at Cheyenne.Grove@osumc.edu, 614-293-9587 or ALSResearch@osumc.edu.

Other helpful resources:

- Northeast ALS Consortium - register for their webinars to learn more about current events, research and other helpful information.
 - <https://www.neals.org/>
- ALS Untangled - <http://www.alsuntangled.com/>
- Answer ALS - <https://www.answers4als.org/>

- I AM ALS and ALS Signal: Clinical Research Dashboard - To move promising treatments forward until we find cures.
 - <https://iamals.org/alsignal/>
 - iamals.org/navigation - Connect with an I AM ALS Navigator to get personalized support through every step of ALS.
- **ALS Office Hours Project** - this is a group of caring people with various lived experiences with ALS, and professional expertise in the field of ALS. The Office Hours Zoom Link is open every Friday from 1pm – 2pm ET. No appointment is needed, and there are no costs or fees. The website to access the Zoom Link: officehoursals.com.
- National Institute of Health
 - <http://www.nih.gov/health/clinicaltrials>
 - <https://clinicaltrials.gov/>
- Muscular Dystrophy Association (MDA)
 - <https://www.mda.org/disease/amyotrophic-lateral-clerosis/research/special-programs>
- ALS Association (ALSA)
 - <http://www.alsa.org/research/>



Integrating Research into the Clinic

- Open and active listening
- Discuss goals and expectations for trials
 - From trial perspective
 - From patient perspective
- Be mindful of the ALS Clock



Preparing for a Clinical Trial:

- Medical Records including the following:
 - Clinic notes from last neurologist office visit
 - This often will include the most recent ALSFRS-R scores
 - Any genetic test results
 - Results of pulmonary function testing



Things to think about:

- Selecting a trial
 - How long will the trial last?
 - Generally around 26 weeks (6 months)
 - What is the medication and its mechanism of action?
 - Talk with your doctor
 - What route: oral, IV, subcutaneous, intrathecal
- Commitments to the study
 - Time (length and frequency of the visits)
 - Tests

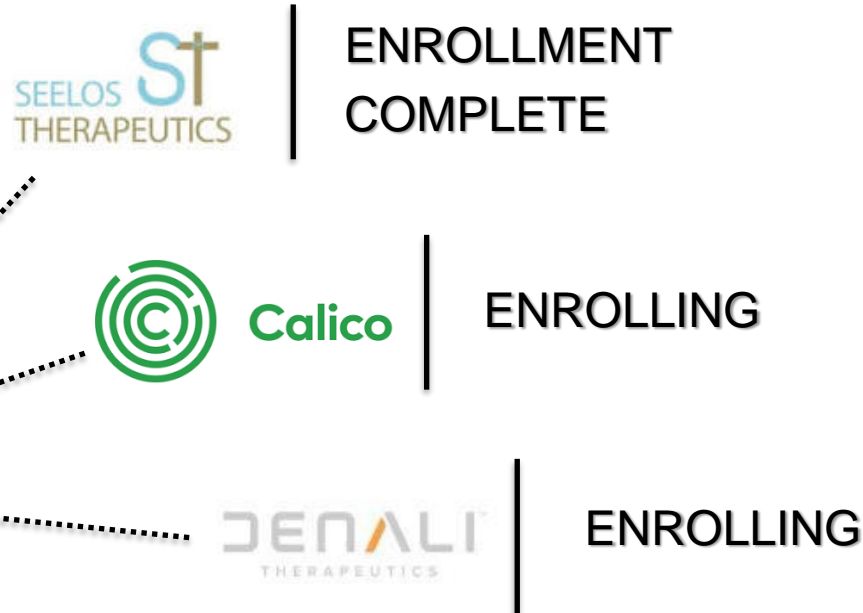
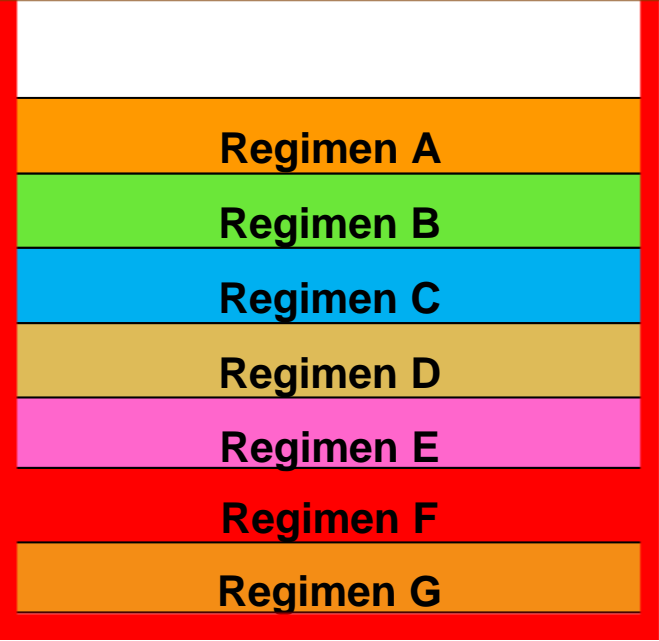
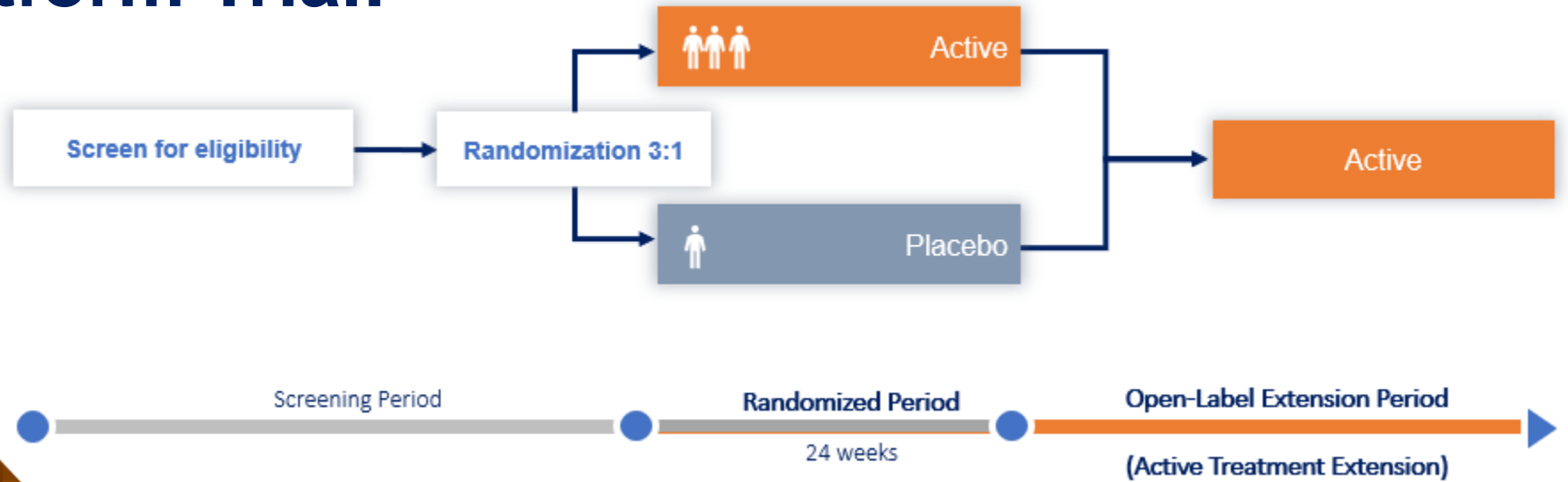
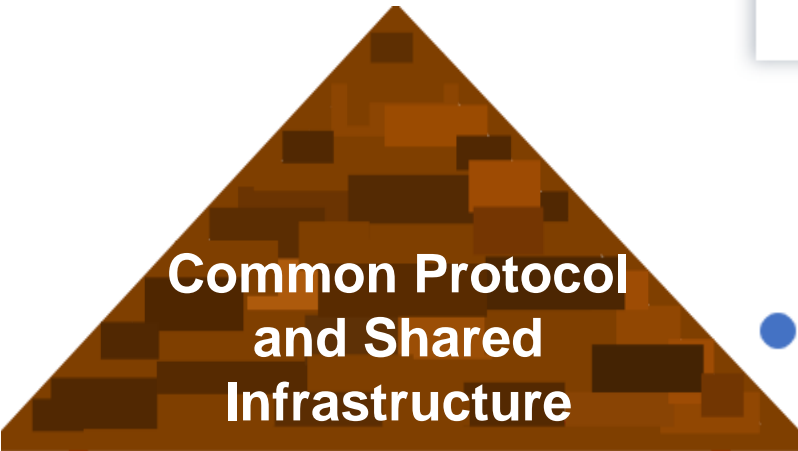




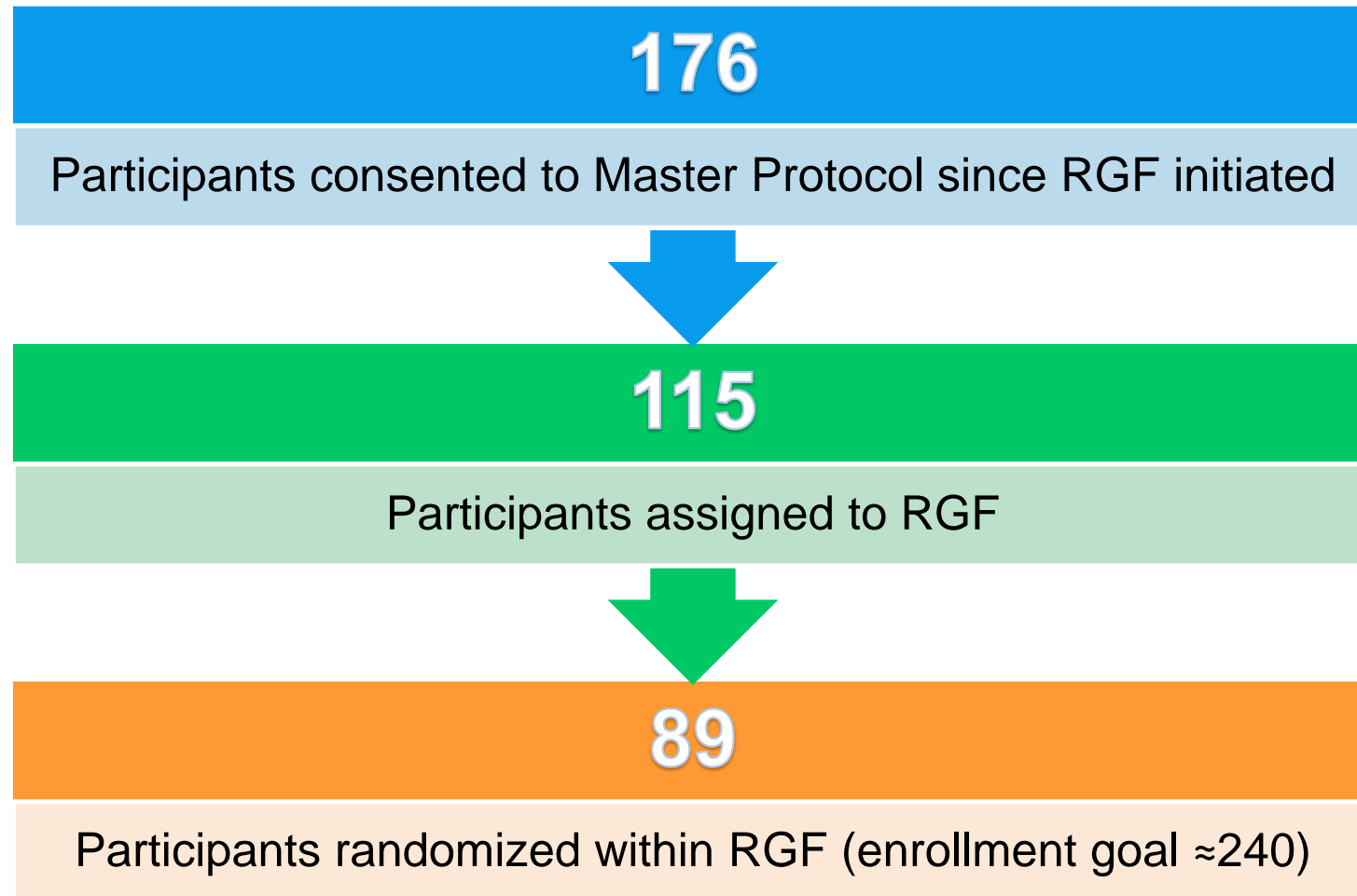
The sunflower is a symbol of strength, grace and dignity. When in clusters, the flowers support each other, allowing for growth in a multitude of shapes, sizes and colors. This diversity represents the people fighting this disease and reminds us to always have hope.



HEALEY ALS Platform Trial:



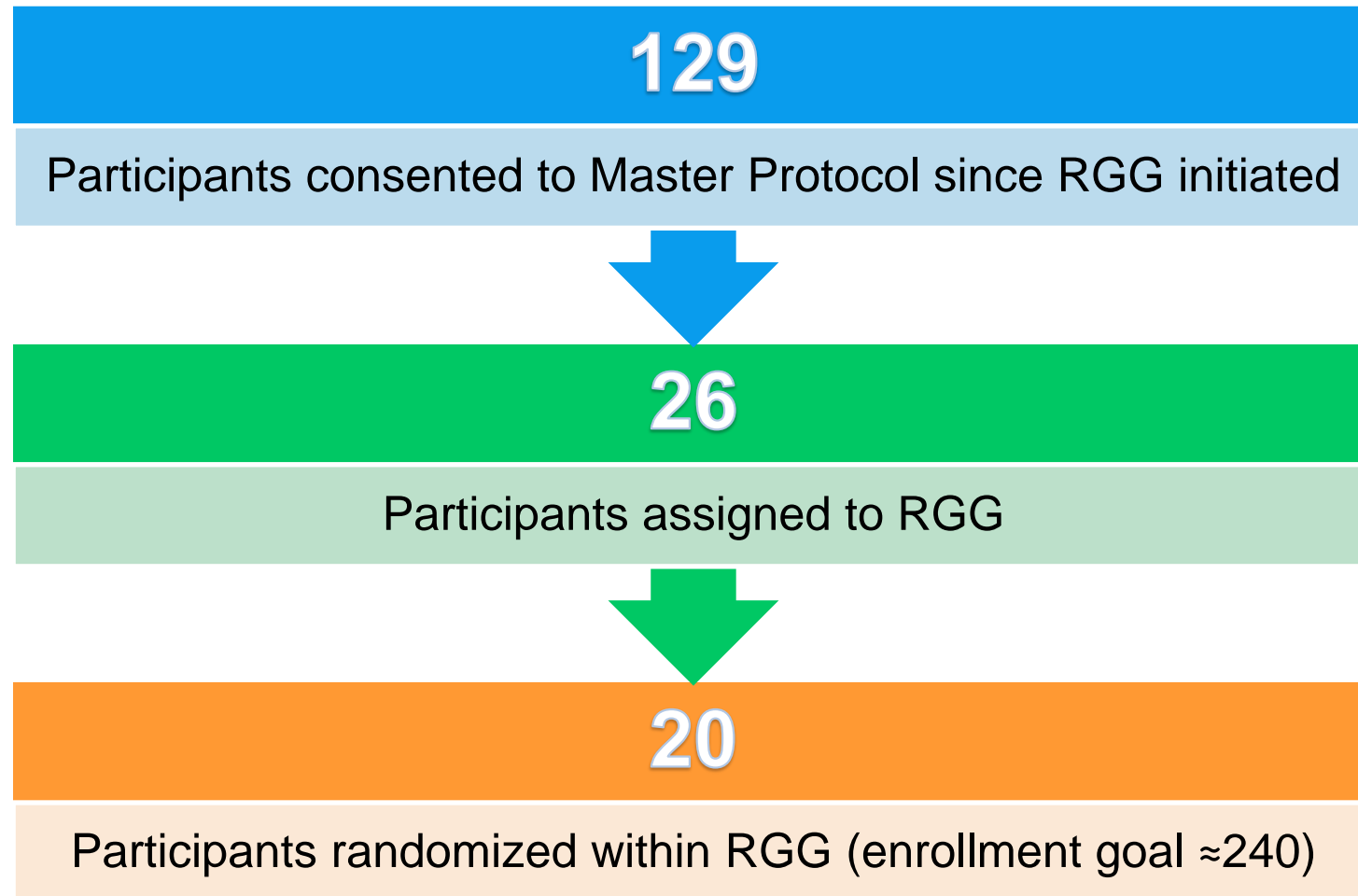
Enrollment Update: Regimen F (as of 7/13/23)



Thank You

for your partnership in ALS research

Enrollment Update: Regimen G (as of 7/13/23)



Thank You

for your partnership in ALS research

Checking Site Status Online

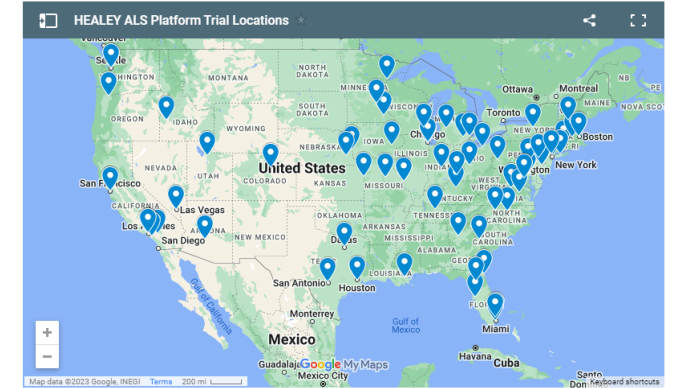
Contact a study team near you to discuss enrollment opportunities

List of Participating Sites

Sites marked "Recruiting" are currently enrolling participants. Sites marked "Active, Not recruiting" are active in the Platform Trial (for example, they are following participants in ongoing regimens that have already completed enrollment) but are not enrolling new participants at this time.

Site	State	Enrollment Status	Trial Contact Information
Barrow Neurological Institute	AZ	Recruiting	Whitney Dailey
Cedars-Sinai Medical Center	CA	Recruiting	Sophia Mostowy
Forbes Norris MDA/ALS Research Center, California Pacific Medical Center	CA	Recruiting	Teji Dulai
Kaiser Permanente, Los Angeles Medical Center	CA	Recruiting	Mary H. Berganza

Map of Participating Sites



<https://bit.ly/3g2NZr5>

Regimen G Drug Science Q&A Webinar



Open to everyone!
Thursday, July 20th
5:00-6:00pm Eastern

Topic: DNL343 Drug Science and Mechanism of Action

Link to Register: <https://bit.ly/3NqJU1j>

DEALI

Patient Navigation

Central resource for people living with ALS



Catherine Small

Phone: 833-425-8257 (HALT ALS)

E-mail: healeyalsplatform@mgh.harvard.edu

Weekly webinar
registration:



<https://bit.ly/3r6Nd2L>

ALS Link sign-up:



<https://bit.ly/3o2Ds3m>

Upcoming Webinars:

July 20th- Regimen G / DNL343 Drug Science Q&A Webinar with Denali

August 3rd- with guest host Suma Babu, MBBS, MPH (Mass General Hospital)



Allison Bulat